severe lipid storage phenotype of Wolman disease fibroblasts. Electron microscopy confirmed significant correction of the lysosomal lipid storage in AdhLAL-infected Wolman disease fibroblasts at the ultrastructural level. I.v. injection of AdhLAL into wild-type mice resulted in a 13.5-fold increase in hepatic LAL activity, and overexpression of LAL was not associated with toxic side effects. These data demonstrate high-level lysosomal expression of recombinant LAL in vitro and in vivo and show the feasibility of gene therapeutic strategies for the treatment of Wolman disease.

RE.CNT 44 THERE ARE 44 CITED REFERENCES AVAILABLE FOR THIS RECORD
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(FILE 'HOME' ENTERED AT 10:55:57 ON 22 FEB 2007)

FILE 'MEDLINE, CAPLUS, BIOSIS, EMBASE, SCISEARCH' ENTERED AT 10:56:40 ON 22 FEB 2007

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                E GRABOWSKI GREGORY/AU
L1          257 S E2, E3, E4
                E DU HONG/AU
L2          229 S E3
L3          453 S L1 OR L2
L4         2158 S ("LIPID HYDROLYING PROTEIN" OR "LIPID HYDROLASE" OR "LYSOSOMA
L5           46 S L3 AND L4
L6         2563 S "GENE THERAPY" AND "VIRAL VECTOR" AND PLASMID
L7            0 S L4 AND L6
L8        12605 S "GENE THERAPY" AND "VIRAL VECTOR"
L9         2563 S L6 AND L8
L10           0 S L4 AND L8
L11          23 S L4 AND "GENE THERAPY"
L12          63 S L5 OR L11
L13           9 S L12 AND VECTOR
L14           1 S L12 AND PLASMID
L15           1 S L14 AND (LIPOSOME OR "LIPID VESICLE")
L16           0 S L12 AND "VIRAL VECTOR"
L17           9 S L13 OR L14 OR L15
L18           5 DUP REM L17 (4 DUPLICATES REMOVED)
L19          30 DUP REM L12 (33 DUPLICATES REMOVED)
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